Meeting Minutes

Institution:	Case Western Reserve University
Meeting Date:	August 11, 2025
Meeting Time	10:00 AM Eastern Time
Meeting Type:	Virtual Platform Teleconference (Remote) Open to the Public

Members in Attendance:

Member	Voting	Member Type
Hauke, Caitlyn	Yes	Chair: Biosafety Expert/HGT Expert
Rastein, Daniel	Yes	Core Member: Biosafety Expert/HGT Expert
Helm, Allen	Yes	Core Member: Biosafety Expert/HGT Expert
Yun, Yang H.	Yes	Local Unaffiliated Member
Karlo, Colleen	Yes	Site Contact
Young, Andrew	Yes	Biological Safety Officer

Invited Members Not in Attendance:

Member	Voting	Member Type
Wilson, LaKetta	Yes	Local Unaffiliated Member
Tepfenhart, Benjamin	Yes	Site Contact

Guests:	DiFrancesco, Kathryn; Barber, Holly; Tovach, Nicholas
Staff:	Smith, Jennifer

Call to Order: The IBC Chair called the meeting to order at 10:00 AM. A quorum was present as defined in the Sabai IBC Charter.

Conflicts of Interest: The IBC Chair reminded all members present to identify any conflicts of interest (COI). No COI was declared by any voting member of the IBC for any of the items on the agenda.

Public Comments: No public comments were made prior to or at the meeting.

Review of Prior Business: None

Previous Meeting Minutes: None

New Business:

PI:	Sandhu, Naemat
Sponsor:	Ultragenyx Pharmaceutical Inc.
Protocol:	UX701-CL301 A Phase 3, Randomized, Double-blind, Placebo-controlled Study of Adeno-associated Virus Serotype 8-mediated Gene Transfer of Glucose-6-phosphatase in Patients with Glycogen Storage Disease Type Ia
Review Type:	Initial Review
NIH Guidelines Section:	III-C-1

Trial Summary: UX701-CL301 is a first-in-human Phase I/II/III clinical trial sponsored by Ultragenyx Pharmaceutical Inc. and designed to assess the safety and efficacy of UX701 in adult participants with Wilson Disease. UX701 is a recombinant, replication-defective adeno-associated virus (AAV) vector designed to express a truncated human ATP7B protein to restore functional ATPB7 in the body. The investigational product (IP) is administered by administered by intravenous infusion.

Biosafety Containment Level (BSL): The study agent UX701 consists of an AAV vector which does not contain any hazardous transgenes which can be classified as a Risk Group 1 (RG1) agent for which Biosafety Level 1 (BSL-1) may be considered. [redacted] therefore the AAV may be classified as an RG2 agent for which Biosafety Level 2 (BSL-2) is the recommended containment level. The administration of this agent in a clinical setting further requires compliance with the OSHA Bloodborne Pathogen Standard (*29 CFR 1910.1030*).

Risk Assessment and Discussion:

- The Committee reviewed the clinical trial Sponsor's study documents and the Sabai-generated comprehensive study-specific Risk Assessment which collectively provided a thorough description of the recombinant or synthetic nucleic acid molecules (investigational product/s) and the proposed clinical research activities involving the IP.
    - In summary, the primary risks in this clinical trial include potential occupational exposure from accidental spills, splashes, and needlestick exposures of the IP during preparation and/or administration procedures. These potential risks are mitigated through a combination of relevant staff training, safe clinical practices (including Standard Precautions and sharps safety) and use of appropriate PPE (as prescribed in the Risk Assessment and documented in the IBC submission package).

- The Site confirmed that only study personnel who have been educated on the potential biohazards and the precautions to be taken when working with the IP will handle the IP or any materials contaminated by the IP.
- The Site confirmed that study personnel are sufficiently trained in the practices and techniques required to safely work with the IP.
- The Site confirmed that staff members receive Bloodborne Pathogens training.
- Occupational Health Recommendations: None
- [redacted] the IBC determined that BSL2 containment would be more appropriate for this study. The Site had no concerns.

- The Committee reviewed the Site's facility details, relevant study-specific procedures and practices, the PI's credentials, and other applicable information provided by the Site for the purposes of the IBC review.
  - The Site verified that the information provided by the Chair was accurate.
  - The Biohazard Sign will be administratively updated to reflect work at BSL2 and define AAV.
  - The Committee noted that a disinfectant was not visible in the photo of the spill kit in the Biohazard Waste Storage area. The Committee reminded the Site to have appropriate disinfectants and absorbent materials in the areas where a spill may occur. The Site had no concerns.

Motion: A motion of Full Approval for the study at BSL-2 was passed by unanimous vote. There were no votes against and no abstentions.

- Contingencies stated by the Committee: None
- Stipulations stated by the Committee: None

Review of Incidents: Nothing to report.

IBC Training: Nothing to report.

Reminder of IBC Approval Requirements.

Adjournment: The IBC Chair adjourned the meeting at 10:33 AM

Post-Meeting/Pre-Approval Note: None